Gennrich, Jane - Medicaid

From: Eide, Tamara J. - Medicaid
Sent: Thursday, April 28, 2016 9:45 AM
To: Gennrich, Jane - Medicaid
Subject: FW: CF Foundation Comments to the Idaho Pharmacy and Therapeutics Committee
Attachments: ID - P&T Letter - Inhaled Antibiotics, Orkambi, & Kalydeco - May 2016.pdf

Tami Eide, Pharm.D., BCPS
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From: Parker, Stephanie [mailto:stparker@cff.org]
Sent: Wednesday, April 27, 2016 1:33 PM
To: Eide, Tamara J. - Medicaid
Cc: Feng, Lisa; Erdo, Jackie
Subject: CF Foundation Comments to the Idaho Pharmacy and Therapeutics Committee

Dear Dr. Eide,

Please find attached a written submission from the Cystic Fibrosis Foundation for consideration by Idaho Medicaid and the Pharmacy and Therapeutics Committee. Thank you, please consider us a resource now and in the future.

Best,

Stephanie Parker
Coordinator, Access Policy and Innovation
Cystic Fibrosis Foundation
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CYSTIC FIBROSIS
FOUNDATION
ADDING TOMORROWS

April 27, 2016

Tami Eide, PharmD
Idaho Medicaid P&T Committee
3232 Elder Street
Boise, Idaho 83705

Dear Dr. Eide and Members of the Pharmacy and Therapeutics Committee:

On behalf of people in Idaho living with cystic fibrosis (CF), we write to urge Idaho Medicaid to include coverage for ivacaftor (Kalydeco®), lumacaftor/ivacaftor (Orkambi®), and FDA-approved inhaled antibiotics designed for the treatment of CF on the preferred drug list (PDL).

About the CF Foundation
Cystic fibrosis is caused by genetic mutations that result in the malfunction of a protein known as the cystic fibrosis transmembrane conductance regulator (*CFTR*). Decreased *CFTR* function causes irreversible damage and the associated symptoms of cystic fibrosis and leads to early death, usually by respiratory failure. As the world's leader in the search for a cure for CF and an organization dedicated to ensuring access to high quality, specialized CF care, the Cystic Fibrosis Foundation accredits 120 care centers, including 2 in Idaho, and 55 affiliate programs nationally that provide multidisciplinary, patient-centered care in accordance with systematically reviewed, data-driven clinical practice guidelines. Treatment options for this rare, life-threatening disease are extremely limited.

About Ivacaftor (Kalydeco) and Lumacaftor/Ivacaftor (Orkambi)
People with cystic fibrosis have a fundamental medical need for increased *CFTR* protein function. Kalydeco is the only FDA-approved medication that improves the function of *CFTR* for individuals with certain mutations including G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, G1349D, and R117H while Orkambi improves function for individuals with two copies of the F508del mutation.

These modulating therapies for CF patients have been shown to immediately improve airway surface liquid properties, reduce airway obstruction, and improve deficiencies in non-respiratory organ systems. Evidence show significant improvements in lung function (FEV_1), as well as trends indicating reductions in the rate of pulmonary exacerbations, increased body mass index (BMI), and improvement in patient-reported respiratory outcomes (CFQ-R). The totality of efficacy evidence is indicative of overall benefit.

Initiating treatment with modulators in patients with the indicated *CFTR* gene mutation earlier in the disease progression process helps to ensure patients have the greatest potential for overall lifetime benefit. Appropriate treatment has the potential to slow the progressive decline in health and prevent permanent, irreversible organ damage (lung, pancreas, etc.) characteristic of cystic fibrosis. It is not medically reasonable or responsible to withhold an effective treatment until the patient suffers an irreversible decline in health and loss of lung function.

About Inhaled Antibiotic Therapies

Inhaled antibiotics are used to improve respiratory symptoms in people with cystic fibrosis who have *Pseudomonas aeruginosa*, a bacterium that colonizes in the lungs and is associated with increased morbidity and mortality in people with this disease. Use of CF specific antibiotics has been shown to decrease *P. aeruginosa* in sputum and improve lung function and quality of life.[1,2,3]

Due to increasing antibiotic resistance in this intensely treated population, antibiotic options are limited. Each FDA-approved inhaled antibiotic therapy represents an important component to the CF treatment arsenal. Further, some patients are not tolerant of one or more therapies due to drug toxicity or difficulty administering the product. Therefore, providers should have discretion to prescribe the most effective CF-specific inhaled antibiotic for individual patients.

For many patients, continuous alternating therapy with several antibiotics is vital to suppressing *P. aeruginosa* and other pathogens and maintaining lung function. Continuous alternating therapy entails the use of a second inhaled antibiotic during the off-month for Cayston®; Cayston® can only be used for a 28-day course before the patient must be off the drug for another 28 days. This period without an inhaled antibiotic treatment can lead to pulmonary exacerbations and a decline in lung function. Access to a tobramycin product allows patients on Cayston® to have FDA-approved alternatives during the 28-day off-regimen period to prevent a decline in health status during this period. Alternating or combining these antibiotic therapies may be helpful in suppressing chronic infection.

Policy Recommendations

Per FDA approval, the CF Foundation recommends Idaho Medicaid make Kalydeco available to individuals with on-label mutations age 2 years and older and Orkambi available to all CF patients age 12 years and older with two copies of the F508del mutation. Restricting access to these life-saving therapies could result in severe and avoidable health consequences for CF patients.

Additionally, limiting patient choice for inhaled antibiotics can greatly impact the health of people with cystic fibrosis who require inhaled antibiotics for optimal treatment. These therapies are an important part of standard CF care. We urge you to provide access to all inhaled antibiotic therapies for people with CF as prescribed by their physician.

Please contact Jackie Erdo, MPH, Manager of Access Policy and Innovation, at jerdo@cff.org or 301-841-2628 with any further questions. We look forward to working with you on this important issue.

Sincerely,

Bruce C. Marshall, MD
Senior Vice President of Clinical Affairs

Lisa Feng, MPH
Senior Director, Access Policy & Innovation

1. Retsch-Bogart GZ, Quittner AL, Gibson RL, Oermann CM, McCoy KS, Montgomery AB, Cooper PJ. Efficacy and safety of inhaled aztreonam lysine for airway *Pseudomonas* in cystic fibrosis. *Chest* 2009;135:1223-32.
2. Ramsey BW, Pepe MS, Quan JM, Otto KL, Montgomery AB, Williams-Warren J, Vasiljev KM, Borowitz D, Bowman CM, Marshall BC, *et al.* Intermittent administration of inhaled tobramycin in patients with cystic fibrosis. Cystic Fibrosis Inhaled Tobramycin Study Group. *N Engl J Med* 1999;340:23-30.
3. Quittner AL, Buu A. Effects of tobramycin solution for inhalation on global ratings of quality of life in patients with cystic fibrosis and *Pseudomonas aeruginosa* infection. *Pediatr Pulmonol* 2002;33:269-276.